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Miltenyi Biomedicine

Industry perspectives on the ATMP landscape and future trends

EUCOPE and Miltenyi Biotec

EPTRI General Assembly – Bologna – 14/03/2025

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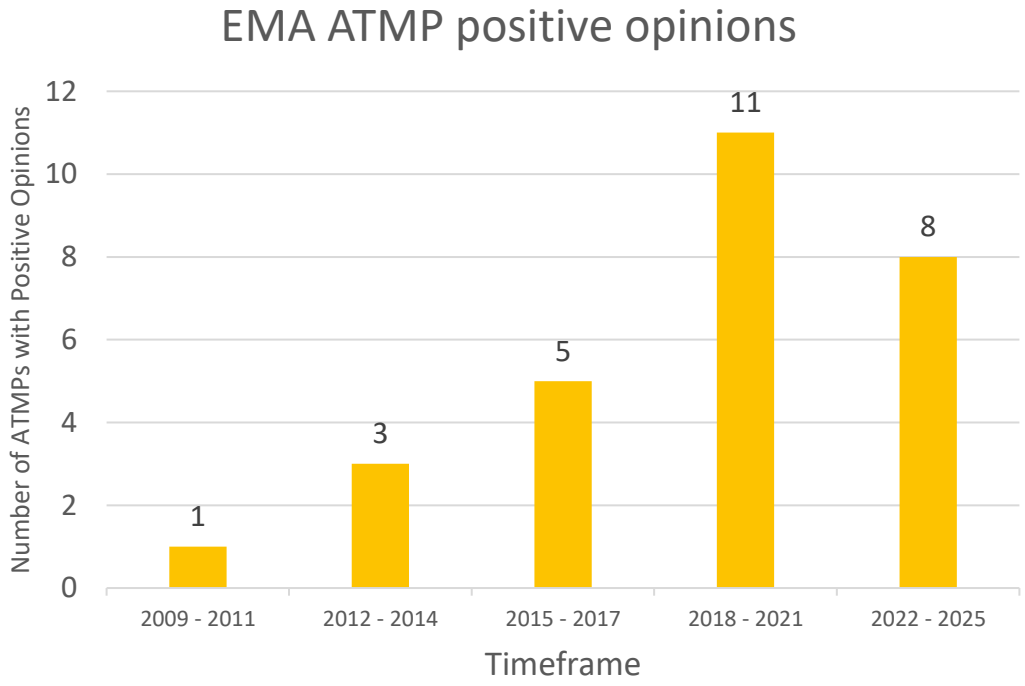
Healthcare policy trends

- Developers remain dedicated to the development of ATMPs – however it takes place in waves
- Despite decades of research, uptake of ATMPs remains in the early stages
  - **Decisions and priority setting by healthcare systems** and regulatory authorities will have a significant
- Decisions in recent months and years have sent a positive signal to ATMP developers
- Former FDA Commissioner Gottleib in 2019 – “by 2025, we predict that the FDA will be approving 10 to 20 cell and gene therapy products a year”



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Evolving European Policy Environment

Equal access

Affordability

Availability

are major priorities for the current European Commission and Member States.

Identifying and implementing **appropriate reimbursement frameworks** has been a major challenge to the uptake and continued interest in ATMP investment

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Political Priorities

European leadership has been in early-stage development and discovery

- Clear drop-off in the number of clinical trials in the EU – driven by a host of factors, including regulatory barriers
- Impact of the **General Pharmaceutical Legislation** is still to be seen

- New Commission has placed a renewed focus on **<u>competitiveness & innovation</u>** – opportunity for political signals (i.e. platform technologies)
  - 2024 Draghi report identified ATMPs as a strategic and priority sector for investment by private and public institutions
  - 2025/2026 will see Commission's proposals on the Biotech Act and Life Science Strategy which are expected to place emphasis on ATMPs

- Developers and healthcare systems are still looking for viable commercialisation models
  - Need ensure healthcare system readiness as number ATMPs grows

- Opportunity to create mutually reinforcing and virtuous cycles

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EU policy puzzle – Policy challenges for CGTs

Regulatory policy

Market access policy

Competitiveness

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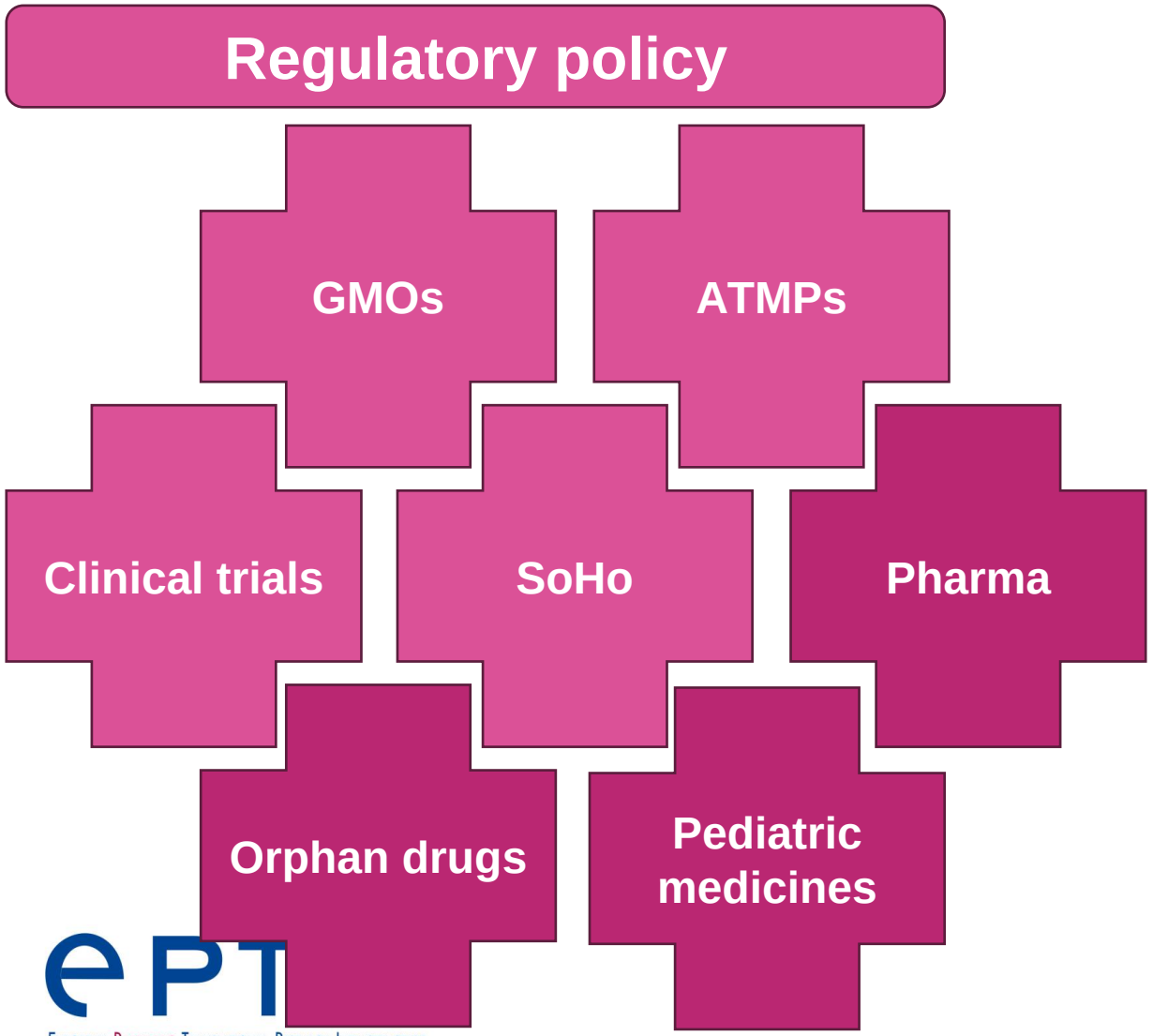
EU policy puzzle – Policy challenges for CGTs



COMPLEXITY, UNPREDICTABILITY, RED TAPE

- **Many different legislations apply**
- **Many different issues/challenges,** for example:
  - **GMO requirements applying to ATMPs** (Contained Use Directive and the Deliberate Release Directive)
  - **Borderline products between SoHo and ATMPs** – unclear classification and unclear role of EMA
  - **Etc.**

Pharmaceutical legislation review:

- Timelines (180 days vs. 210 days)
- Expanded PRIME eligibility
- Revised EMA structure
- Modulation of incentives
- (high) unmet medical needs
- **Regulatory sandboxes**
- **Decentralized manufacturing**
- **Environmental GMO issues**
- **Hospital exemption**

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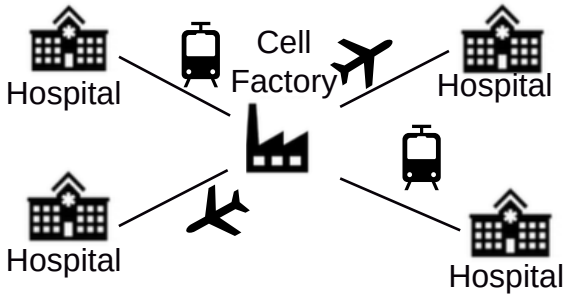
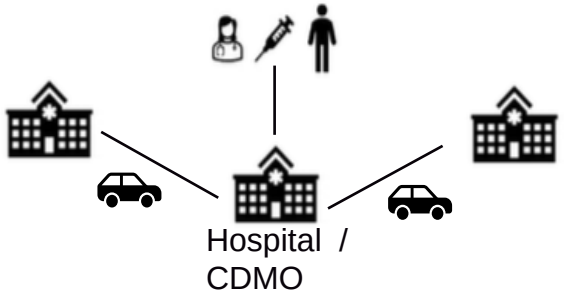
Revision of the EU Pharmaceutical framework

Decentralized manufacturing (Art 144-53, Dir).

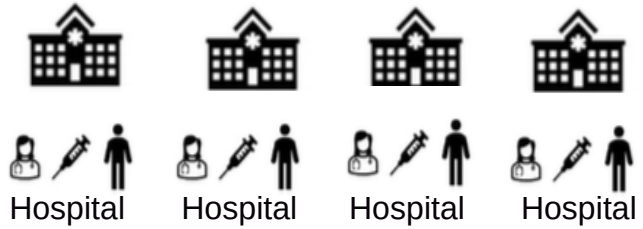
- **Recognition that manufacturing or testing of ATMPs may need to take place in sites close to patients**
- **Limits the obligation to obtain a manufacturing authorisation only to the central site**



Centralised Manufacturing



Decentralised Manufacturing



Point of Care Manufacturing

Regulatory sandboxes (Article 113, Reg)

- **New regulatory path to test** and create innovative medicinal **products and technologies**
- **When no other pathway exist** or if not possible to develop the medicinal product or category of products within the existing requirements applicable to medicinal products due to challenges arising from characteristics related to the product development. Could be used to test **point-of-care manufacturing**.

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Revision of the EU Pharmaceutical framework

GMO requirements (Art. 5a (new), Reg)

- **Simplification and harmonization** of the environmental risk assessment (ERA) in terms of identifying and evaluating potential adverse events of genetically modified organisms.
- **No systematic exemption of ATMPs** from GMO requirements. However, **some Articles of the GMO legislation* shall not apply to medicinal products containing or consisting of genetically modified organisms**:
  - this includes operations related to the *supply and clinical use*, including the *packaging and labelling*, distribution, storage, transport, preparation for administration, *administration, destruction or disposal*.

(*) Contained Use Directive and Deliberate Release Directive

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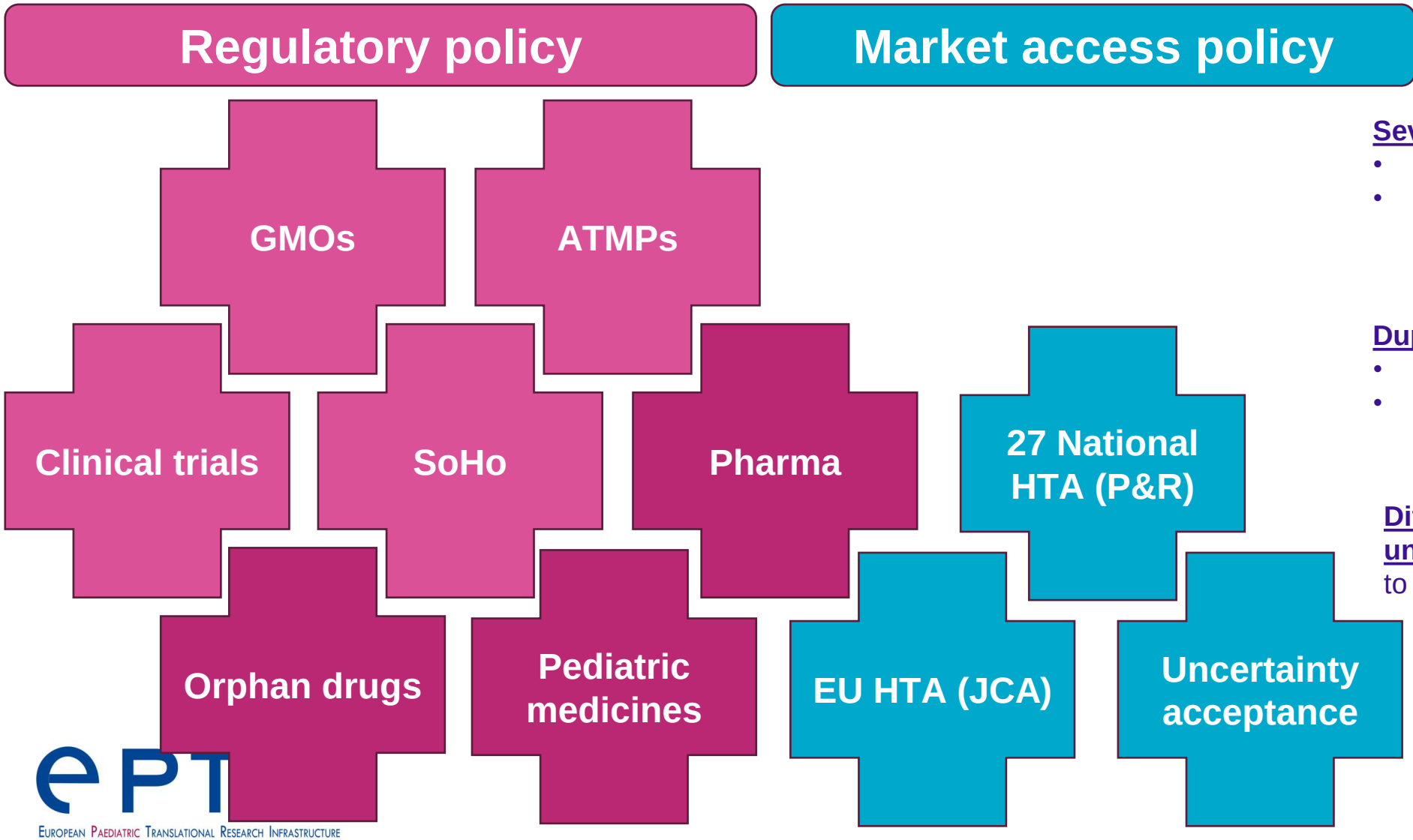
EU policy puzzle – Policy challenges for CGTs



Several levels of (HTA) assessments:

- EU: Joint clinical assessment (JCA)
- National: additional national economic assessments and P&R negotiations

Duplication and discrepancies

- between EU / national requirements
- between regulators, payers and HTA data requirements

Different degrees of acceptation of uncertainty, e.g. RWD/RWE*, vs. head to head comparisons

*Real World Data/Evidence

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EU Joint Clinical Assessment (JCA)
Overview

Background

HTA definition

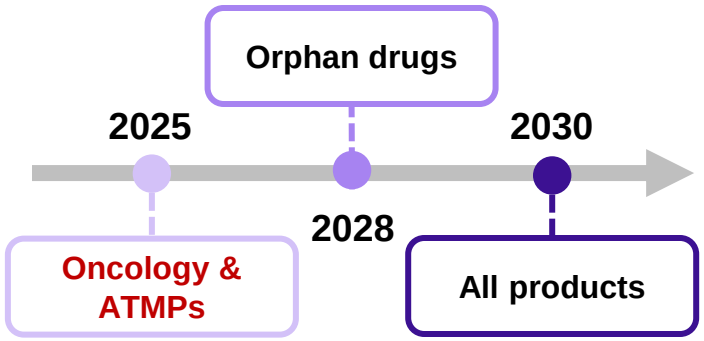
Health Technology Assessment (HTA): the systematic evaluation of one health technology, **in comparison to alternative treatments**

JCA definition

EU JCA is a joint framework incorporating common methodology and approach for **clinical assessments and scientific consultations, on the EU level**

Timelines

EU JCA roadmap



Challenging timeline

Once the final scope is received, health technology developers will have **only 100 days to prepare** the dossier under standard procedure, or 60 days under accelerated procedure

Evidence

PICO scope

Under the identified **Population, Intervention, Comparison, Outcome (PICO)** scope, evidence for comparison between the health technology and alternative therapies will be submitted

Direct vs indirect comparison

Both direct and indirect comparison will be allowed in JCA.

Direct evidence is the golden standard

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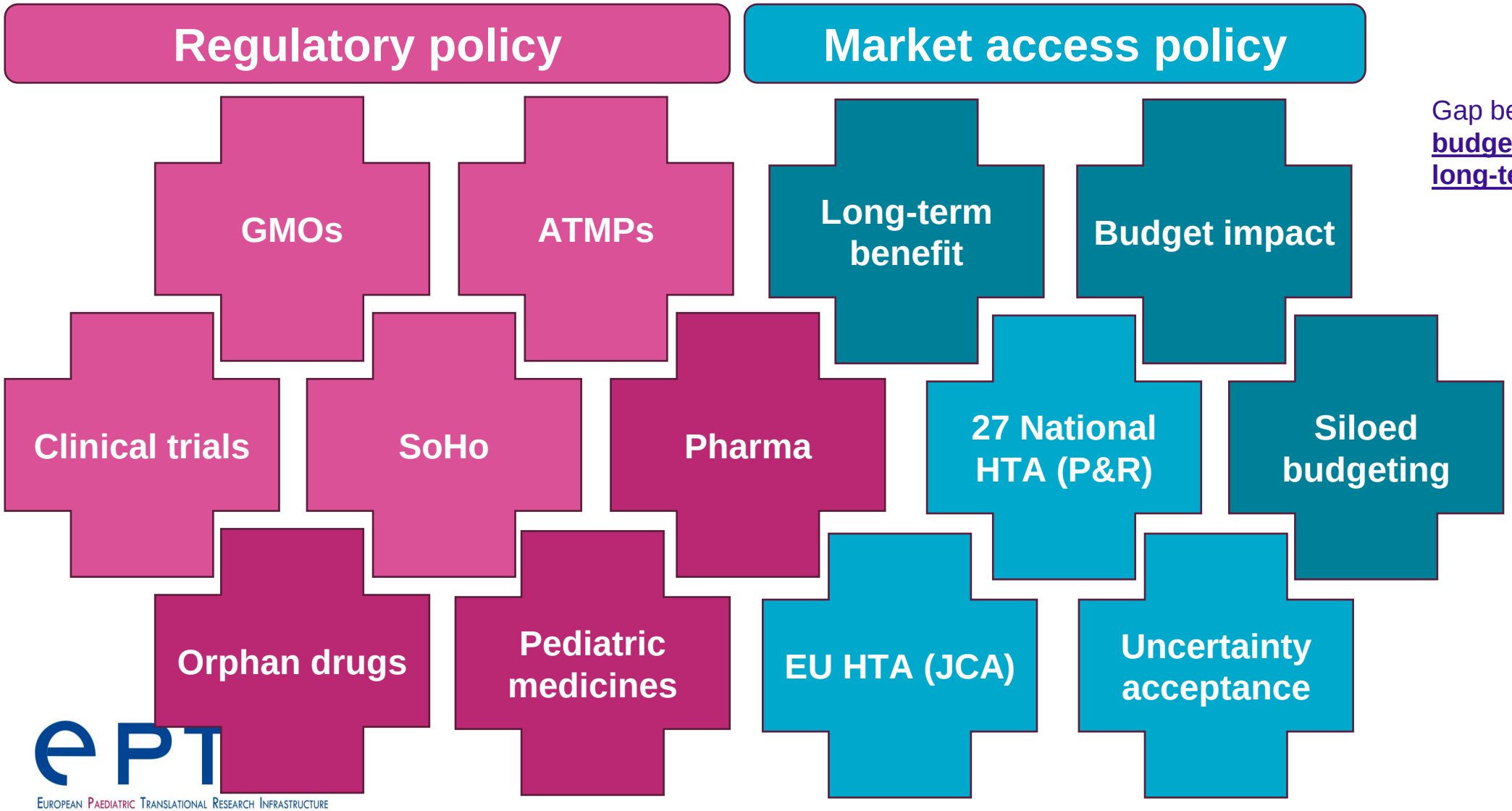

Gap between **short-term siloed budgeting and assessment of long-term value of CGTs**

Not all countries take account of:

- **Long term benefit of CGTs**
- **Indirect cost-savings,** notably when looking at **budget impact**

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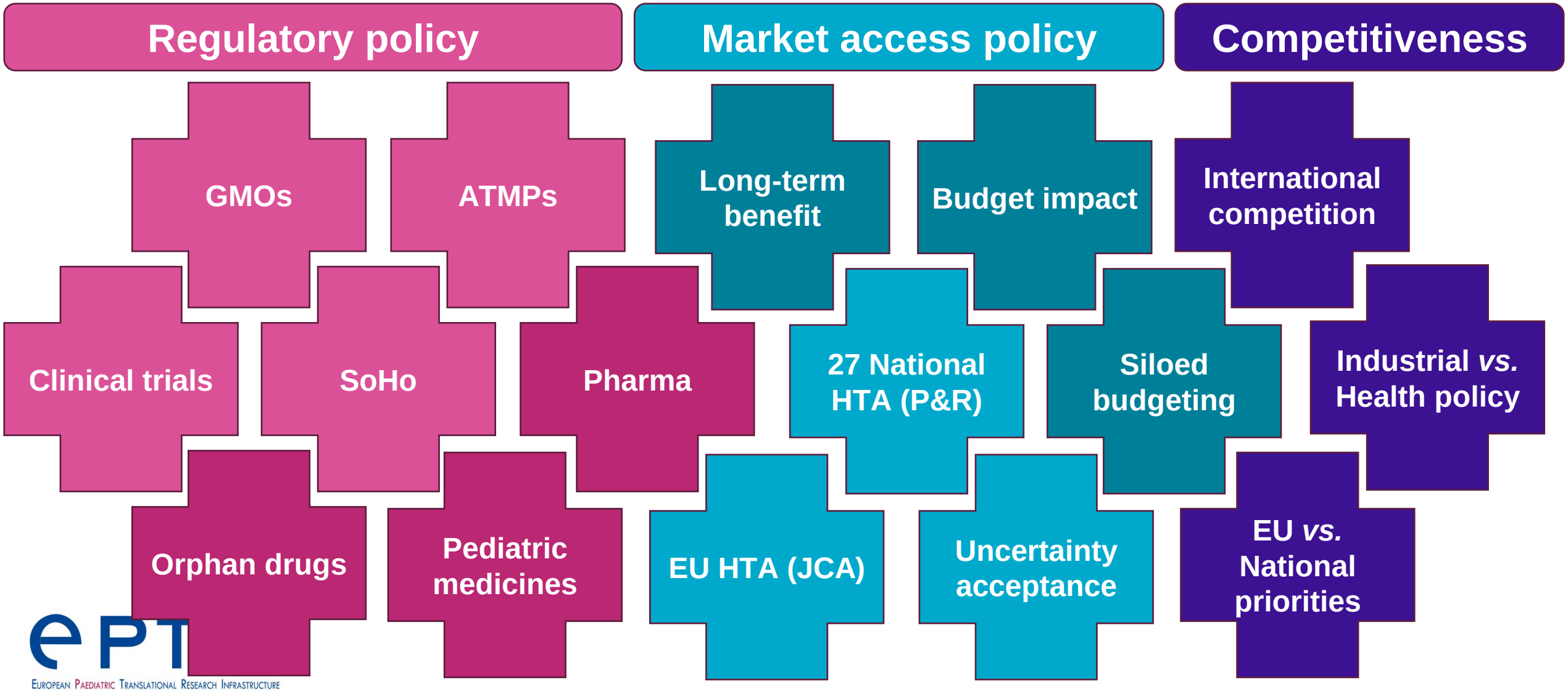

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Strategic health priorities for the new mandate (2024-2029)

Biotech Act

Life Science Strategy

Critical Medicines Act

Simplification agenda

Medical Device Regulation / In-vitro Medical Device Regulation

Disease Area Action Plans

Data Union Strategy

EU Competitiveness Compass

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Thank you!

Any Questions?

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